CASE STUDY

Two Paediatric studies in Rare Endocrine-Metabolic Disease

Background

Leading global pharmaceutical company.

Study 1:
Phase III, randomised, double-blind, placebo controlled, **Endocrine/ Metabolic study** in **Heterozygous Familial Hypercholesterolemia**.

Study 2:
An **Open-Label s**tudy to evaluate the **Efficacy** and **Safety** in **Children** and **Adolescents** with **Homozygous Familial Hypercholesterolemia.**

Engagement

- Full Service

Challenge

- Limited Patient Pool - challenge in identifying paediatric patients with this rare metabolic condition
- Involving a patient from a country where the study was not conducted
- Complex regulatory pathways

Optimapharm solution

- Rigorous selection of Principal Investigator
- Experienced CRA
- Proactive approach to achieve high engagement
- Close collaboration with study site

Outcomes

Study 1: Heterozygous Familial Hypercholesterolemia Study

First EU patient enrolled

100% recruitment achieved

Study 2: Homozygous Familial Hypercholesterolemia Study

100% recruitment achieved